CIRM Funded Clinical Trials

Phase 1 Study of Autologous CD4LVFOXP3 in Participants with IPEX Syndrome

Disease Area:	IPEX Syndrome
Investigator:	Rosa Bacchetta
Institution:	Stanford University
CIRM Grant:	CLIN2-13259 (Pre-Active)
Award Value:	$11,999,179
Trial Sponsor:	Stanford University
Trial Stage:	Phase 1
Trial Status:	Recruiting
Targeted Enrollment:	36
ClinicalTrials.gov ID:	NCT05241444

Rosa Bacchetta

Details:
IPEX syndrome is a rare condition where the body can't control or restrain an immune response, so the person's immune cells attack their own healthy tissue. The syndrome mostly affects boys, is diagnosed in the first year of life and is often fatal. Children born with IPEX syndrome have abnormalities in the FOXP3 gene. This gene controls the production of a type of immune cell called a T Regulatory or Treg cell. Without a normal FOXP3 +Treg cells other immune cells attack the body leading to the development of IPEX syndrome, Type 1 diabetes, severe eczema, damage to the small intestines and kidneys and failure to thrive.

Dr. Rosa Bacchetta and her team at Stanford University have developed a therapy using the patient's own natural CD4 T cells that, in the lab, have been genetically modified to express the FoxP3 gene and converted into Treg cells. Those cells are then re-infused into the patient with a goal of determining if this approach is both safe and beneficial. Because the cells come from the patients there will be fewer concerns about the need for immunosuppressive treatment to stop the body rejecting the cells. It will also help avoid the problems of finding a healthy donor and graft vs host disease.

Design:
Open label, non-randomized

Goal:
This first-in-human, Phase 1 clinical trial will test the feasibility of the manufacturing and the safety of the administration of CD4^LVFOXP3 in up to 36 human participants with IPEX and evaluate the impact of the CD4^LVFOXP3 infusion on the disease.

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